STEM CELL GENE THERAPY FOR SICKLE CELL DISEASE

Grant Award Details

STEM CELL GENE THERAPY FOR SICKLE CELL DISEASE

Grant Type: Disease Team Planning

Grant Number: DT1-00701

Investigator:

Name:	Donald Kohn
Institution:	Children's Hospital of Los Angeles
Type:	PI

Disease Focus: Sickle Cell Disease, Blood Disorders

Award Value: $12,131

Status: Closed

Grant Application Details

Application Title: STEM CELL GENE THERAPY FOR SICKLE CELL DISEASE

Public Abstract:

Sickle cell disease (SCD), which results from an inherited mutation in the hemoglobin gene that causes red blood cells to “sickle” under conditions of low oxygen, occurs with a frequency of 1/500 African-Americans, and is also common in Hispanic-Americans, who comprise up to 5% of SCD patients in California. The median survival based on 1991 national data was 42 years for males and 48 years for females. Recent data indicate that the median survival for {REDACTED} California patients is only 36 years, suggesting that serious problems regarding access to care exist in this community. By twenty years of age, about 15% of children with SCD suffer major strokes and by 40 years of age, almost half of the patients have had central nervous system damage leading to significant cognitive dysfunction. These patients suffer significant damage to lungs and kidneys as well as severe chronic pain that impacts on quality of life. While current medical therapies for SCD can make a significant difference in short-term effects, the progressive deterioration in organ function results in increased mortality and decreased quality of life. Bone marrow transplant (BMT) from a healthy donor as a source of new blood-forming (“hematopoietic”) stem cells can benefit patients with SCD, by providing a source for life-long production of normal red blood cells. However, BMT is limited by the availability of well-matched donors and the problems that arise from immune reactions between the cells of the donor and the patient. Thus, despite major improvements in clinical care, SCD continues to be a significant cause of morbidity and early mortality. The central hypothesis of this Disease Team Initiative is that using a patient’s own hematopoietic stem cells which are corrected in the hemoglobin gene using gene therapy has the potential to permanently cure this debilitating and common illness with significantly less toxicity than with a BMT from another person. Methods to add a normal copy of the hemoglobin gene to the stem cells from a patient’s own bone marrow stem cells have been developed. A clinical trial using stem cell gene therapy for patients with SCD will be developed and performed by this Team. Our group has world-leading experts in stem cell gene therapy and the care of children with sickle cell disease. Successful use of stem cell gene therapy for sickle cell disease has the potential to provide a more effective and safe treatment for this disease.

Statement of Benefit to California:

Development of methods for regenerative medicine using genetically corrected human stem cells will result in novel, effective therapies that improve the health for millions of Californians and tens of millions of people world-wide. Sickle cell disease is an inherited disease of the red blood cells that results from a specific gene mutation. Sickle cell disease disproportionately afflicts poor minority patients in the State of California, causing severe morbidity, early mortality and high medical costs. Successful treatment of sickle cell disease using gene therapy and blood forming “hematopoietic” stem cells may provide a clinically beneficial way to treat sickle cell disease with greater safety than current options. The clinical trial to be performed will treat sickle cell patients from across the state.
